Clinical Translation of hESC-derived protein therapy that positively regulates the regenerative capacity of post-natal muscle for treating DM1

Grant Award Details

Clinical Translation of hESC-derived protein therapy that positively regulates the regenerative capacity of post-natal muscle for treating DM1

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-12890

Project Objective: The objective of this award is to conduct a well-prepared pre-IND meeting with the FDA for a recombinant human signaling protein-based therapeutic (JUV-161) for restoring muscle strength and function in degenerative muscle disease.

Investigator:

Name:	Jeremy O'Connell
Institution:	Juvena Therapeutics Inc
Type:	PI

Disease Focus: Muscular Dystrophy, Skeletal/Smooth Muscle disorders

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $3,906,376

Status: Pre-Active

Grant Application Details

Application Title: Clinical Translation of hESC-derived protein therapy that positively regulates the regenerative capacity of post-natal muscle for treating DM1

Public Abstract:

Translational Candidate

We engineered a human embryonic stem cell-secreted signaling protein into a biologic for treatment of skeletal muscle disorders.

Area of Impact

Skeletal muscle disorders (including DM1 and sarcopenia) remain major unmet needs that require treatments restoring muscle strength and function.

Mechanism of Action

Our animal data demonstrate an endocrine stimulation by our biologic of endogenous muscle precursor cells restoring muscle stem/precursor survival and differentiation, counters muscle atrophy, and improves muscle strength/endurance in multiple disease models. This process is impaired in age-related and degenerative skeletal muscle diseases including myotonic dystrophy type 1 and sarcopenia.

Unmet Medical Need

Myotonic Dystrophy type 1 (DM1) is the most common muscular dystrophy in adults, affecting an estimated 1 in 2,532 people in the US, slowly depriving them of their ability to walk, use their hands, and breathe- and yet this population is without treatment options.

Project Objective

Pre-IND meeting

Major Proposed Activities

- Develop of GMP compatible manufacturing process and non-GMP production of the biologic
- Qualification of assays for manufacturing process, release potency, and pre-clinical studies
- Perform pre-clincal toxicology, biodistribution, safety, potency, and efficacy

Statement of Benefit to California:

DM1 is the most common muscular dystrophy in adults, affecting an estimated 1 in 2,532 people in California, slowly depriving them of their ability to walk, use their hands, and breathe. California has the largest population of persons with DM1 of any state, and is home to the Myotonic Dystrophy Foundation representing and building support for the needs of the thousands of Californians severely medically and financially impacted by this debilitating disease.
